Get Real

Real-Life Data in Drug Development

Effectiveness Research: Navigating Real World Solutions

WP1 webinar

12 – 1pm CET (11 – Noon UK)

Live from NICE in London

March 23rd, 2016

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Presenters

Sarah Garner
Associate Director Science Policy and Research, National Institute for Health and Care Excellence (NICE)

Pall Jonsson
Senior Scientific Adviser, National Institute for Health and Care Excellence (NICE)

Rob Thwaites
Senior Director, Takeda

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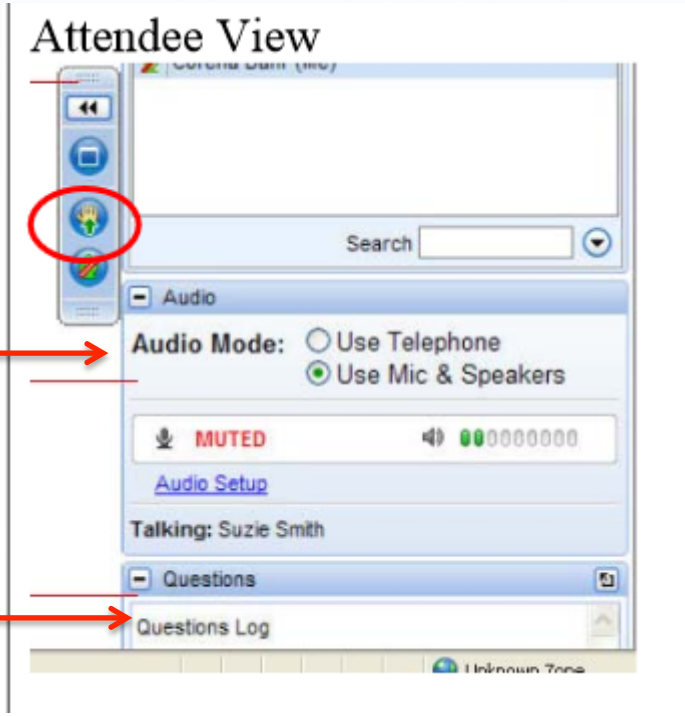
How to Use the Webinar Tools

Raise your hand

Dial in or headphones

Ask a question



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- **CONFERENCE: 17 June 2016 - London**:
  GetReal Putting Real World Healthcare Data to Work
  (Upon invitation only)
- **www.imi-getreal.eu or vitaltransformation.com**

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Effectiveness Research: Navigating Real-world Approaches

Sarah Garner

Establishing relative effectiveness of new drugs

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Effectiveness: The 'fourth hurdle'

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Data paradigms

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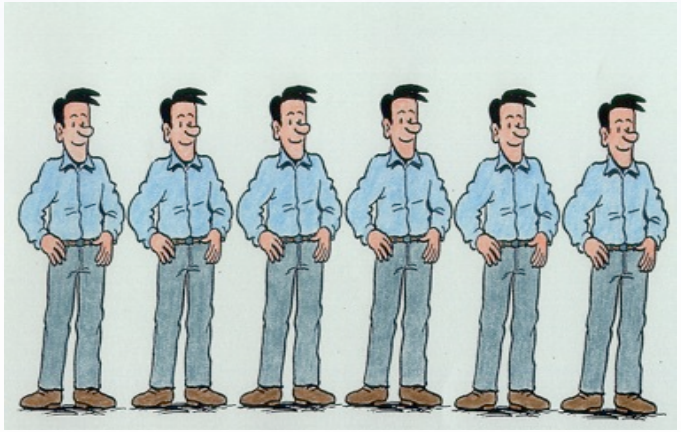
Efficacy *vs* Effectiveness



- 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" - variability and biases

'Effectiveness Challenges'

- Phase III trials too short to capture relevant effects, need to use models:
  Considerable uncertainty in RWE predictions
- RWE likely to be influenced by factors (adherence etc.) not captured in Phase III, model-based estimates unreliable:
  RWE biased?
- Phase III patient population poor fit for local population/general care received may not reflect care in HTA country:
  RWE biased?
- Phase III patient population too broad/poor fit to care pathway (? targeting of therapy):
  Uncertainty in RWE for target sub-populations
- Phase III comparator not appropriate for local HTA: indirect meta-analysis (for RWE) not robust:
  No credible RWE estimate
- Phase III trial event rates for comparator not in line with available RW evidence for comparator:
  RWE biased?

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GetReal: delivering efficient fourth hurdle solutions

- Shared understanding of the technical and process issues from each perspective
- In-depth exploration of 5 challenging disease areas to highlight the issues
- Exploration of novel methodological solutions
- Compilation of best-practice recommendations
- Future research agenda
- Collaboration and trust

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Effectiveness Research: Navigating Real-world Approaches

Pall Jonsson

GetReal: Exploring alternative evidence generation and synthesis

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Innovative Medicines Initiative: *Joining Forces in the Healthcare Sector*

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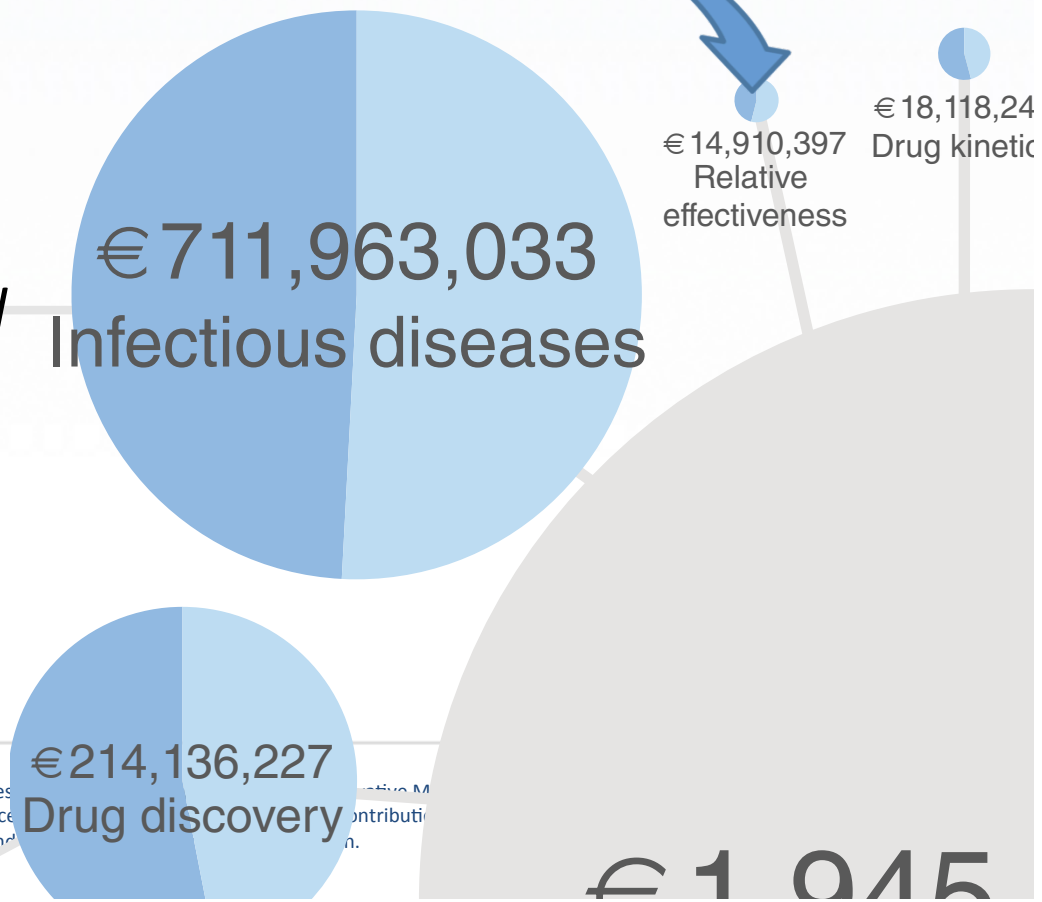
Get Real

Three-year project

" … to better understand how real-world data and analytical techniques can be used to improve the relevance of knowledge generated during development, e.g., through innovation in clinical trial design"



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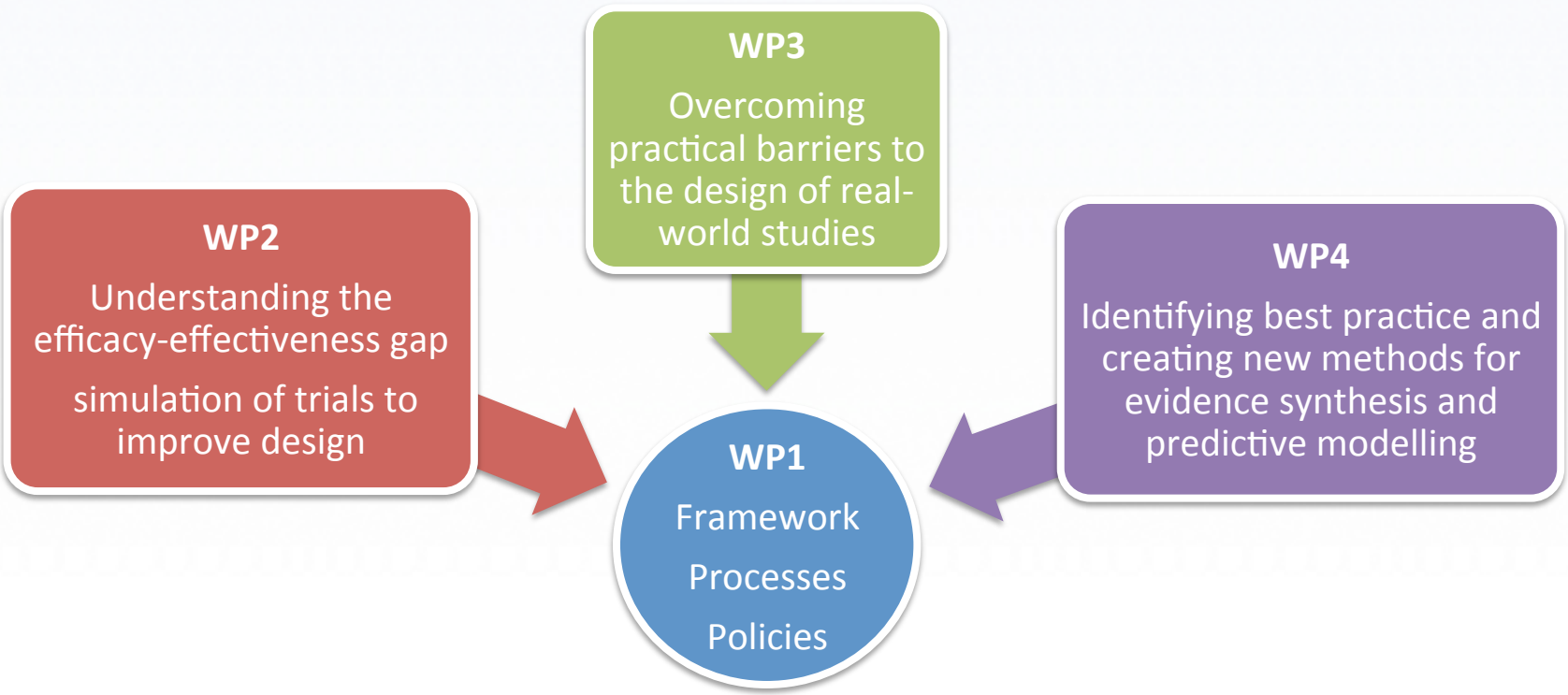
IMI GetReal



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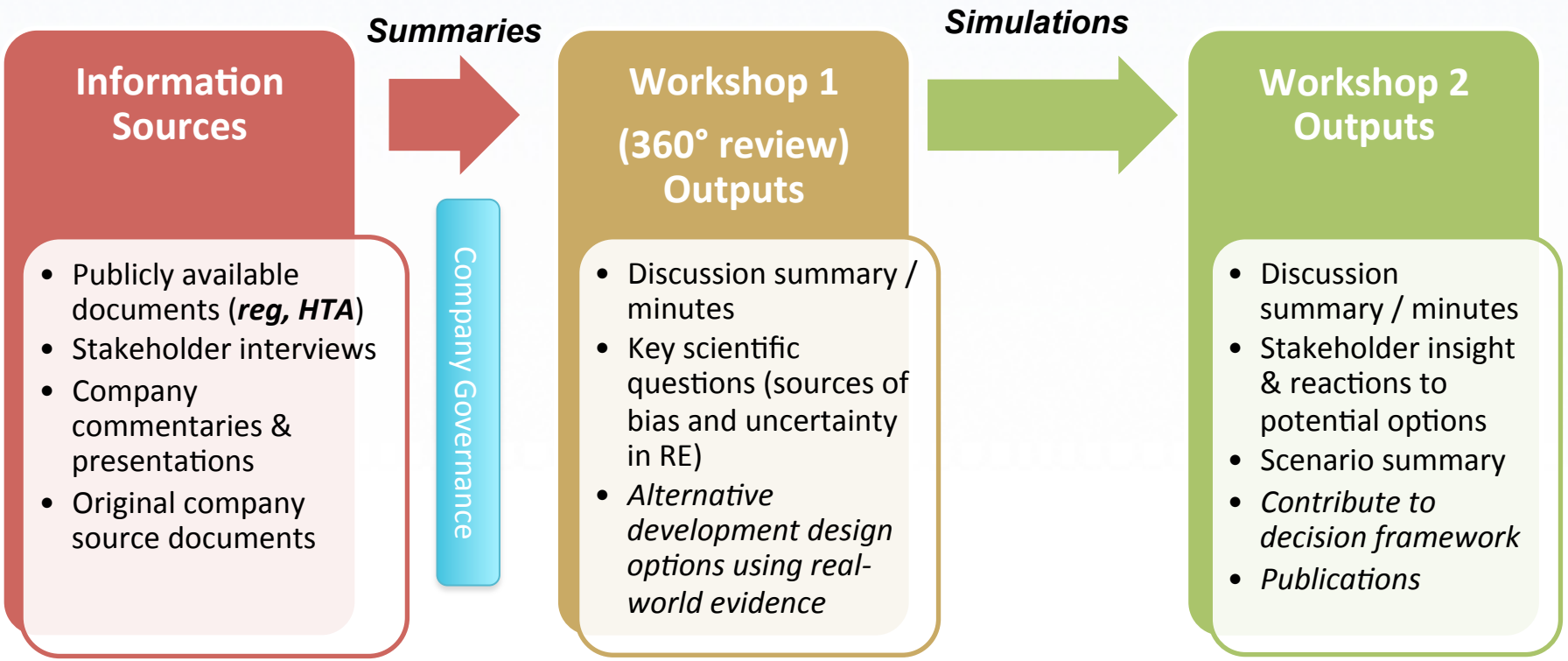
WP1 Case Studies: Redesigning the Development Pathway



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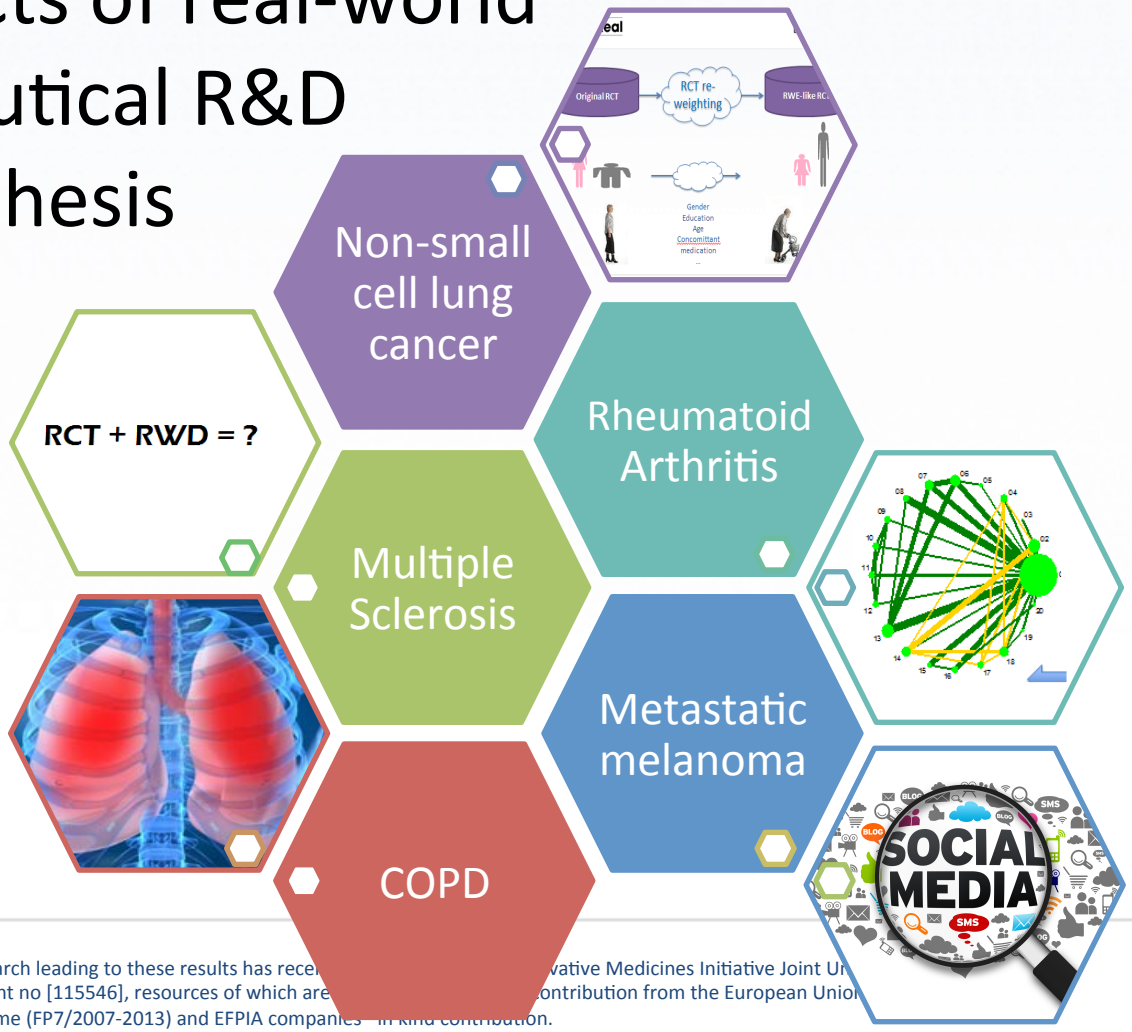
WP1 Case Studies

Highlighting aspects of real-world data in pharmaceutical R&D and evidence synthesis



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GetReal Case Studies: Building a Supportive Environment

- A unique pan-stakeholder workshop environment with a focus on the use of RWE in medicine development and the subsequent assessment
- Provides a 'safe harbour' environment
  - Stakeholder engagement
  - Removing silos
- Identifying and testing acceptability of robust RWE solutions
  - Alternative evidence plans
  - Alternative evidence synthesis
  - Testing acceptability by stakeholders
  - Exploring usability for decision making

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Effectiveness Research: Navigating Real-world Approaches

Rob Thwaites

Towards a real-world evidence framework

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Generating effectiveness evidence: the GetReal Framework

- HTA agencies and payers have proliferated and become more sophisticated, requiring additional evidence
- With the effectiveness challenges, IMI GetReal is exploring alternative evidence generation pathways
- IMI GetReal is producing approaches to address the effectiveness challenges and a framework to help guide these aspects of medicine development strategy

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Evidence development pathways - today

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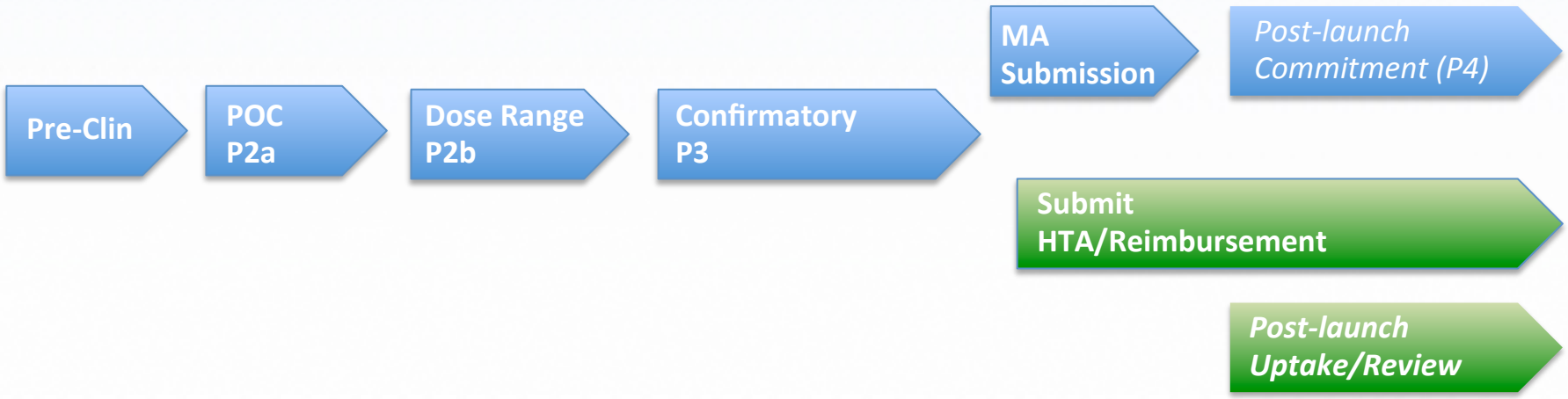
A. Standard pathway for drug development



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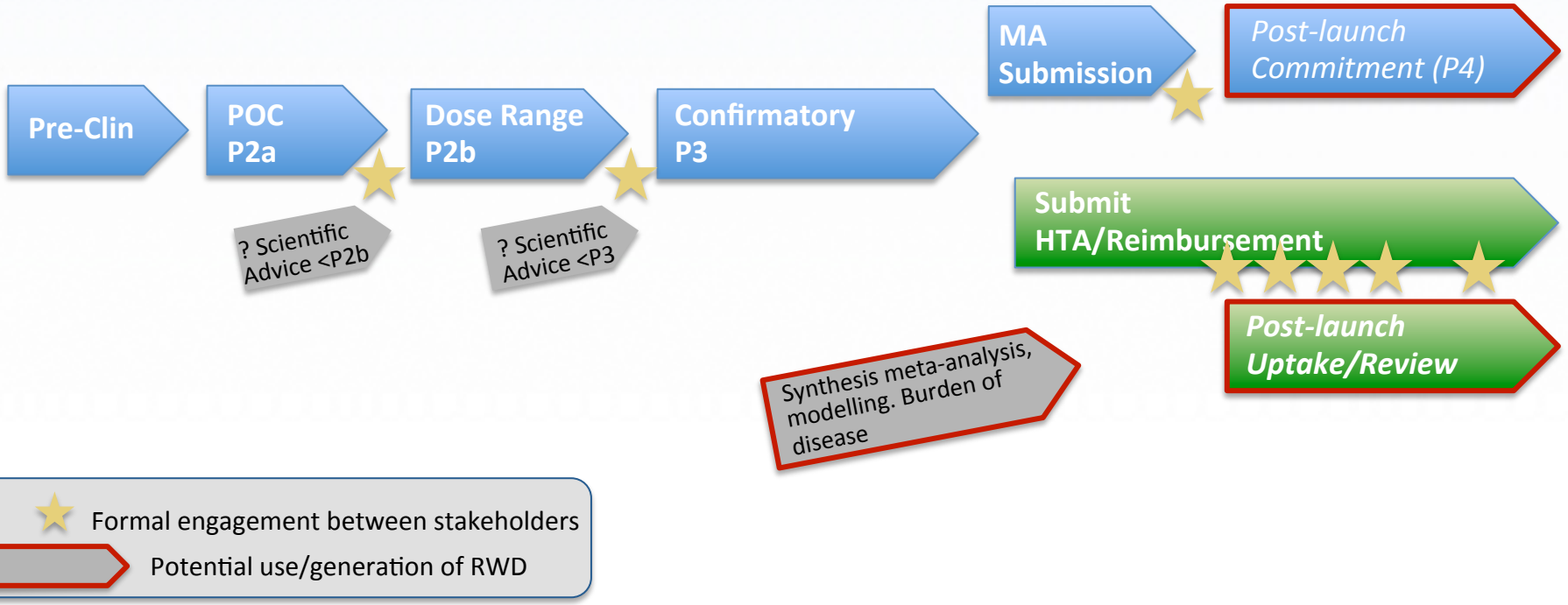

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Effectiveness challenges becoming more common

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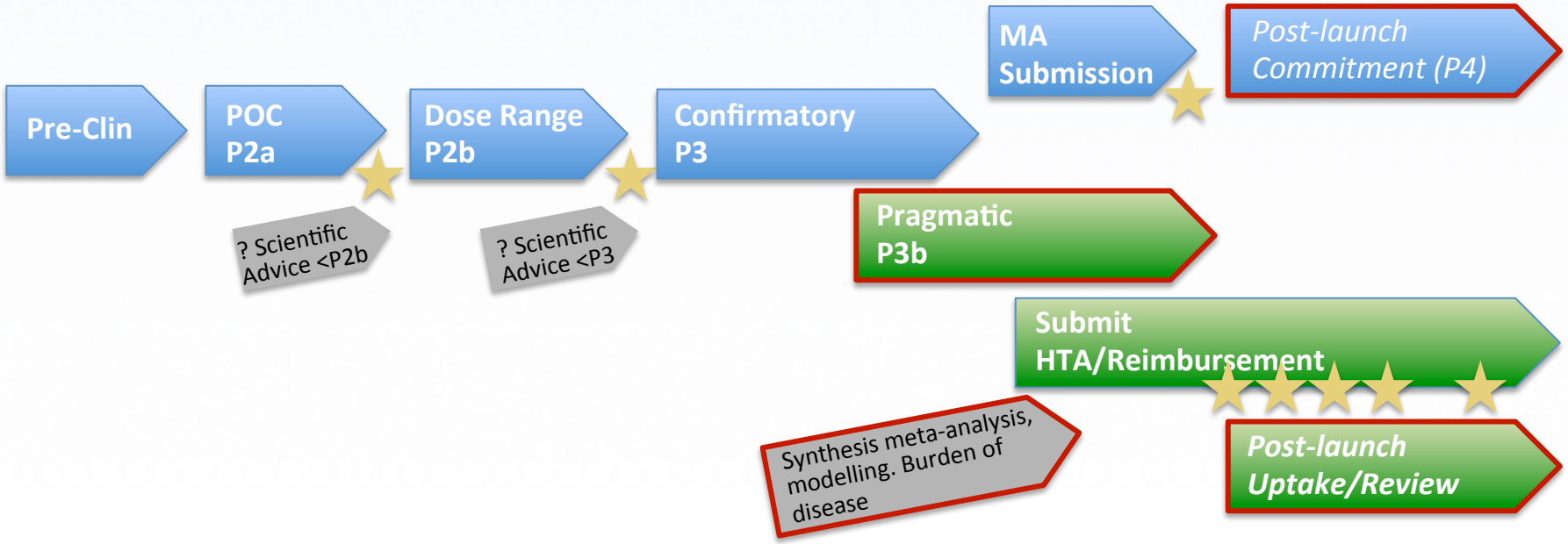
Evidence development pathways – GetReal example

B. Alternative drug development pathway: early PCT example



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Towards a RWE Framework

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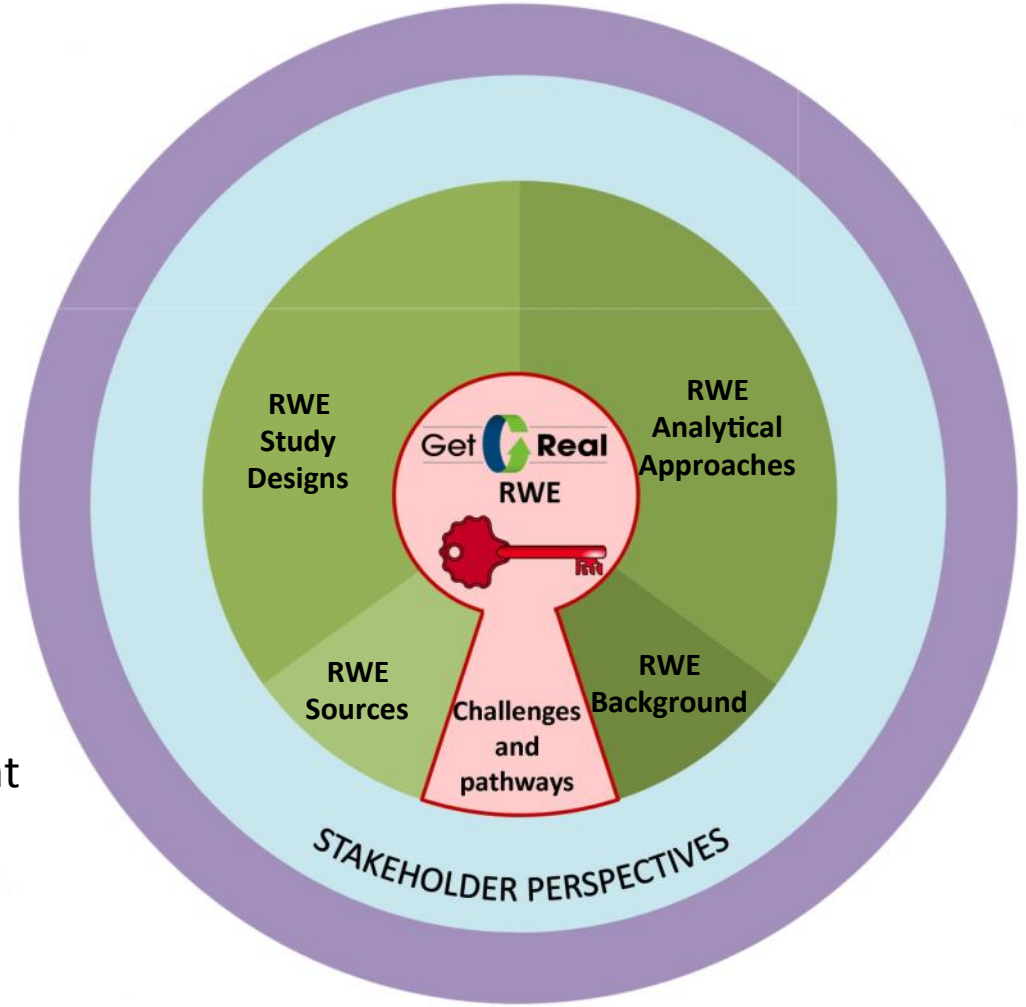
Example

You are coming up to Phase 3 and planning the evidence generation programme:

- Is there a need to generate evidence of effectiveness ?
- Are there drivers of effectiveness that might be "missed" in RCTs?
- How do we get information on *relative* effectiveness?
- Will evidence from this work be relevant and acceptable to physicians and HTA bodies?



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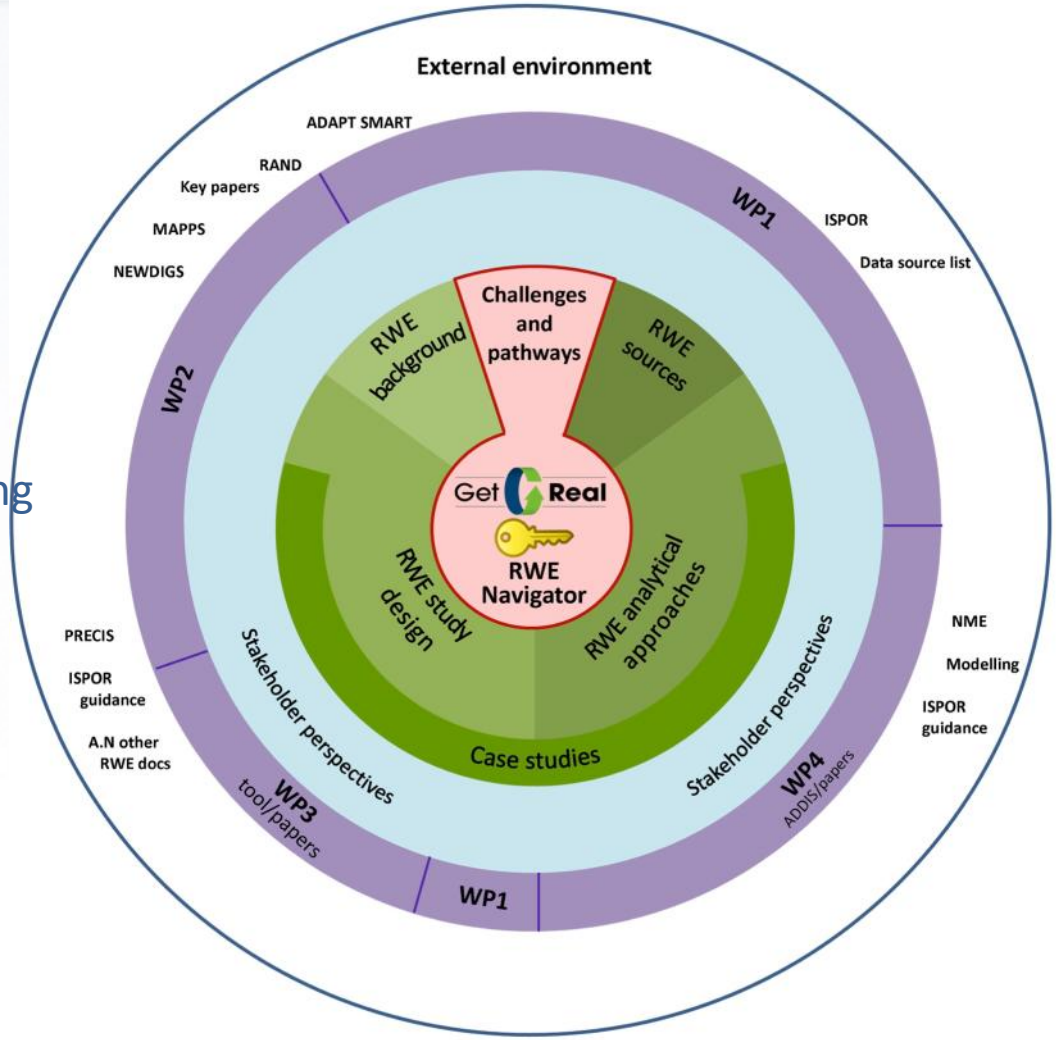
Framework will be the guide to the approaches in GetReal

Real-Life Data in Drug Development

The GetReal RWE Framework

Online platform designed to:

- help guide medicine development strategy: development of evidence of relative effectiveness
- provide insight into options for study designs and analytical approaches using RWD
- guide users towards more detailed material and case studies reported by each GetReal WP
- direct users to authoritative external guidance and sources



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