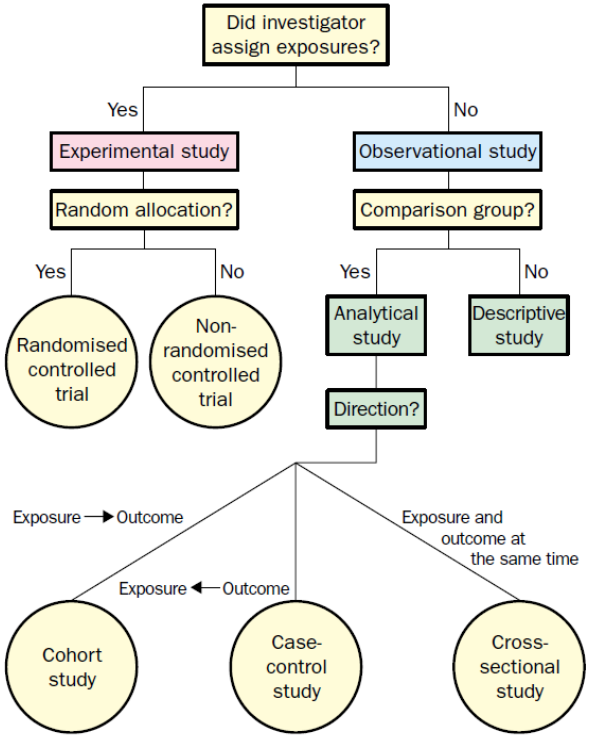
PART I
Fit for purpose:
When and where is real-world evidence most useful?

Some Points to Frame the Discussion

- Types of real world evidence
- Understanding the strengths and limitations of real-world evidence
- Defining purpose and understanding the end user
- Communications

Types of ‘Real World Evidence’

Grimes et al, Lancet, 2002



EVERY study type has strengths and limitations

Hierarchy of Evidence

Oxford Centre for Evidence-Based Medicine, 2002

Level of Evidence	Study Type
Level 1	RCTs
Level 2	Cohort Studies
Level 3	Case-Control Studies
Level 4	Case Series
Level 5	Expert Opinion

In the ‘Real World’….

- Physicians may not use health technologies as they should
  - Off-label use / market expansion
  - Inappropriate use (surgical procedures, inappropriate dosing, etc.)
- Patients may not take health technologies as they should
  - Non-adherence
  - Combinations with interacting drugs / herbal products
  - Device tampering
- Private industry may not promote as they should
  - Promotion of off-label use
  - Aggressive promotion of inappropriate clinical use (e.g. dosing)
- Payers may not reimburse as they should
  - Insufficient coverage / ineffective uptake
  - Restrictive parameters around effective patient populations
  - Brokered ‘backroom deals’

Defining Purpose and Understanding the End User

Despite the valiant efforts of the research group,
the insulin suppository still had one major drawback.

Payers: Key Perspective

- We have a relatively fixed budget
- Healthcare costs seem to keep increasing
- We get many companies with products competing for the same limited ‘pot’
- Why should we fund you (show value)??
- Every dollar we spend on one product we can’t spend on another
- We don’t like risk

Industry Perspective

- We can’t prove ‘real-world’ value without access
- We can’t possibly get ALL the data you need
- Give us a chance to show the value of our product!!
  - Consider innovative PLAs?

Basic Dilemma for the Payer

- Take the data that exists and try and estimate what the ‘real-world’ implications
  - Data from clinical trials show the product CAN work – need to start there but need to compare to current standard of practice
  - Impact on resource utilization can be estimated from this data but won’t be perfect
  - Much of this is a ‘leap of faith’ based on current evidence: small leaps are best

Simplifying Key Needs of Policy-Makers

- Relative to current standard of care, the ideal intervention should:
  - Improve patient outcomes
    - RELEVANT clinical outcomes and quality of life
  - Reduce costs to the healthcare system through decreased healthcare resource utilization OR have marginal costs that are deemed to be ‘acceptable’ for its clinical benefit
  - Have a favorable budget impact OR increase total budget by a marginal amount in line with its anticipated clinical benefit

What Matters to Payers

- Clinical Evidence
  - Outcomes: Effectiveness and Safety
    - Head-to-head comparisons vs indirect comparisons
    - Study design: patient population and follow-up
    - ‘Hard’ outcomes vs surrogate measures
  - Costs
    - Direct vs indirect
  - The ‘intangibles’
    - Patient preference
    - Quality of life: choice of tools

Communications

Panelists

- **Cy Frank**
  - Alberta Innovates Health Solutions President and CEO
- **Greg Zaric**
  - Professor, Richard Ivey School
- **Elaine Campbell**
  - President of AstraZeneca Canada Inc.

Panel Discussion